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**CLINICAL TRIALS IN DOWN SYNDROME FOR CO-OCCURRING CONDITIONS  
ACROSS THE LIFESPAN WORKSHOP**

**AGENDA**

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**Day 1 – May 7, 2020**

- 8:30 a.m.**                      **NIH Welcome**  
Diana W. Bianchi  
*Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD)
- Introduction of Workshop**  
Laurie Ryan, National Institute on Aging (NIA)
- Overview of Workshop Logistics**  
Erika Tarver, NIA
- 8:40 a.m.**                      Prenatal Treatment: Clinical Trial of the Future?  
Diana W. Bianchi, NICHD
- 9:00 a.m.**                      **Session: Pediatric/Developmental Conditions**  
                                 (co-chairs: **Priya Kishnani and Steve Abman**)
- 9:05 a.m.**                      • Pediatric Cardiology Consideration in Children with Down Syndrome  
Ashraf Harahsheh, Children’s National Health System
- 9:20 a.m.**                      • Sleep conditions  
                                 ○ Role of Sleep for Cognitive Development in Down Syndrome  
Jamie Edgin, University of Arizona
- 9:35 a.m.**                      ○ Sleep Apnea in Children with Down Syndrome  
Daniel Combs, University of Arizona
- 9:50 a.m.**                      • Take a Breath: Post-Influenza-Like State in Down Syndrome  
Michael Yeager, University of Colorado Denver
- 10:05 a.m.**                      • Infectious Diseases in People with Down Syndrome  
Andrew Nowalk, University of Pittsburgh
- 10:25 a.m.**                      • JAK Inhibition in Down Syndrome: Treating Autoimmunity and Beyond  
Joaquín Espinosa, University of Colorado Denver
- 10:45 a.m.**                      **BREAK**

- 11:00 a.m. • Leukemia in Children with Down Syndrome: Treading Carefully with Clinical Trials  
Jeffrey Taub, Wayne State University
- 11:15 a.m. • Central Nervous System Conditions  
○ Down Syndrome & ADHD  
Anna Esbensen, Cincinnati Children's Hospital
- 11:30 a.m. ○ Cognitive Considerations for Clinical Trials in Children with Down Syndrome  
Laura Hahn, University of Illinois at Urbana-Champaign
- 11:45 a.m. – 12:15 p.m. LUNCH**
- 12:15 p.m. • Communication and Language Intervention  
○ Speech, language, articulation  
Ann P. Kaiser, Vanderbilt University
- 12:30 p.m. ○ Factors Affecting Listening and Speech Perception in Children with Down syndrome  
Lori Leibold, Boys Town National Research Hospital
- 12:45 p.m. ○ Eye Tracking Technologies to Characterize Visual Attending in Down Syndrome: Implications for Optimizing the Design of Visual Supports for Communication  
Krista Wilkinson, Pennsylvania State University
- 1:00 p.m. – 1:25 p.m. Session Q&A
- 1:25 p.m. Overview of Clinical Trials in the Down Syndrome Population  
Priya Kishnani, Duke University
- 1:45 p.m. – 3:20 p.m. Panel: Considerations for Participation in Clinical Trials – Pediatric and Adult Populations**  
Moderator: Priya Kishnani  
[5-minute presentation per panelist + Q&A]
- Discussants:
- Frank Stephens (self-advocate)
  - Ben Handen (psychologist)
  - Emily Chesnut (parent/advocate)
  - Marilyn Bull (physician)
  - Michelle Sie Whitten (parent/advocate)
  - Florence Lai (physician)
  - David Egan (self-advocate)
  - Kathleen Egan (parent/advocate)
  - Hampus Hillerstrom (parent/advocate)
  - Donna and Wayne Leigh (parent/advocate)
  - Brian Skotko (physician/sibling)

3:20 p.m.

**BREAK**

**Session: Non-pharmacological/Lifestyle Interventions**

**Session Moderator: Annie Cohen**

3:30 p.m.

- Lifestyle and Alzheimer's Disease in Down Syndrome: Physical Activity and Cognitive Stimulation  
Sigan Hartley, University of Wisconsin – Madison

3:45 p.m.

- Music and Social Engagement: Research and Future Directions for Individuals with Down Syndrome  
Miriam Lense, Vanderbilt University

4:00 p.m.

Session Q&A

**Session: Adult/Aging Conditions**

**(co-chairs: Annie Cohen and Mike Rafii)**

4:15 p.m.

- Challenges to Defining Obesity in Down Syndrome  
Andrea Kelly, Children's Hospital of Philadelphia

4:30 p.m.

- Central Nervous System Conditions
  - Alzheimer's Disease, and other Central Nervous System Disorders  
Michael Rafii, University of Southern California

4:45 p.m.

- Regression: A Review of Available Literature and Research Gaps  
Michelle Palumbo, Massachusetts General Hospital

5:00 p.m.

Session Q&A

5:20 p.m.

**Adjourn Day 1 of Workshop**

**Day 2, May 8**

9:00 a.m.

**Session: Overview of INCLUDE Project funded grants on Clinical Trials (R61/R33) and Clinical Trial Readiness (R21)**

Session Moderator: Laurie Ryan

**R21 – Clinical Trial Readiness**

9:05 a.m.

- Epigenetic Silencing of Trisomy HSA21  
Volney Sheen, Beth Israel Deaconess

9:10 a.m.

- Using fNIRS to Study the Neural Correlates of Executive Function and Sleep Impairment in Down Syndrome  
Nancy Raitano Lee, Drexel University

9:15 a.m.

- Acceptability and Performance on In-Home Polysomnography in Youth with Down Syndrome  
Ignacio Tapia, Children's Hospital of Philadelphia

9:20 a.m.

- Measures of Pulmonary Health in Children with Down Syndrome  
Emily DeBoer, University of Colorado Denver

9:25 a.m.

- Early Risk for ADHD Symptoms in Young Children with Down Syndrome  
Deborah Fidler, Colorado State University

9:30 a.m.

- Bronchus-Associated Lymphoid Tissue: Friend or Foe in Down Syndrome  
Michael Yeager, University of Colorado Denver

9:35 a.m.

- Down Syndrome, Early Cataracts, Eye Diseases, and Beta-amyloid Conformers  
Geoffrey Chang, University of California, San Diego

**R61/R33 – Clinical Trials Development**

9:40 a.m.

- Medications for Obstructive Sleep Apnea to Improve Cognition in Children with Down Syndrome (MOSAIC DS)  
Daniel Combs, University of Arizona

9:45 a.m.	<ul style="list-style-type: none"> <li>Evaluating Assessment and Medication Treatment of ADHD in Children with Down Syndrome Anna Esbensen, Cincinnati Children's Hospital</li> </ul>
9:50 a.m.	<ul style="list-style-type: none"> <li>JAK Inhibition in Down Syndrome Joaquín Espinosa, University of Colorado Denver</li> </ul>
9:55 a.m.	<ul style="list-style-type: none"> <li>Clinical trials to Prevent Alzheimer's Disease in Down Syndrome: Trial Ready Cohort – Down syndrome (TRC-DS) Michael Rafii, University of Southern California</li> </ul>
10:00 a.m.	<ul style="list-style-type: none"> <li>Positive Airway Pressure for the Treatment of the Obstructive Sleep Apnea Syndrome in Children with Down Syndrome Ignacio Tapia, Children's Hospital of Philadelphia</li> </ul>
10:15 a.m.	<b>BREAK</b>
10:30 a.m.	<b>Session: Clinical Trial Infrastructure</b> <b>Session Moderator: Steve Abman</b>
10:30 a.m.	<ul style="list-style-type: none"> <li>Down Syndrome Patient Registries Steve Abman, University of Colorado Denver</li> </ul>
10:45 a.m.	<ul style="list-style-type: none"> <li>DS-Connect® Sujata Bardhan, NICHD</li> </ul>
11:00 a.m.	<ul style="list-style-type: none"> <li>PTN-DS: Clinical Pharmacokinetics and Safety Trials in Down Syndrome Mara Becker, Duke University</li> </ul>
11:15 a.m.	<ul style="list-style-type: none"> <li>Pediatric Heart Network Julie Miller, HealthCore</li> </ul>
11:30 a.m.	<ul style="list-style-type: none"> <li>Alzheimer's Clinical Trial Consortium – Down Syndrome (ACTC-DS) Michael Rafii, University of Southern California</li> </ul>
11:45 a.m.	Session Q&A
12:00 p.m. – 12:30 p.m.	<b>LUNCH</b>

<b>12:30 p.m.</b>	<b>Session: Recruitment into Clinical Trials</b> Session Moderator: Mike Rafii
12:30 p.m.	<ul style="list-style-type: none"><li>• Recruitment of Diverse Populations, Lessons Learned and Implications for Down Syndrome Research Annie Cohen, University of Pittsburgh</li></ul>
12:45 p.m.	<ul style="list-style-type: none"><li>• Consent<ul style="list-style-type: none"><li>○ Obtaining Consent into Clinical Trials: State Specific Policies and Requirements Sharon Krinsky-McHale, New York State Institute for Basic Research in Developmental Disabilities</li></ul></li></ul>
1:00 p.m.	<ul style="list-style-type: none"><li>○ Obtaining Consent or Assent Ben Handen, University of Pittsburgh</li></ul>
1:15 p.m.	Sessions Q & A
<b>1:30 p.m.</b>	<b>Report from Break-out Teleconference Sessions</b>
	<ul style="list-style-type: none"><li>• Pediatric Issues and Considerations Steve Abman and Priya Kishnani</li><li>• Adult Issues and Considerations Annie Cohen and Mike Rafii</li></ul>
2:10 p.m.	Breakout Session Reports Q & A
<b>2:30 p.m.</b>	<b>Closing Remarks</b> Gary H. Gibbons National Heart, Lung and Blood Institute
<b>2:45 p.m.</b>	<b>Adjourn Workshop</b>